

CLAIMS:

1. Use of a sphingoid-polyalkylamine conjugate for the preparation of a pharmaceutical composition for the delivery of a nucleic acid molecule into a target cell, wherein said sphingoid-polyalkylamine conjugate comprises a sphingoid backbone carrying, via a carbamoyl bond, at least one polyalkylamine chains.
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2. The use of Claim 1, wherein said nucleic acid molecule has at a physiological pH a net negative dipole moment, at least one area carrying a negative charge or a net negative charge.
3. The use of Claim 2, wherein said nucleic acid molecule is a plasmid DNA.
- 10 4. The use of Claim 2, wherein said nucleic acid molecule is a small interference RNA (siRNA).
5. The use of Claim 2, wherein said nucleic acid molecule is an oligodeoxynucleotide (ODN).
6. The use of Claim 5, wherein said ODN contains at least one CpG motif
15 (CpG-ODN).
7. The use of any one of Claims 1 to 6, wherein said sphingoid-polyalkylamine conjugates forms lipid assemblies.
8. The use of Claim 7 wherein said sphingoid-polyalkylamine conjugate forms micelles and/or vesicles.
- 20 9. The use of any one of Claims 1 to 8, wherein the sphingoid backbone is selected from ceramide, dihydroceramide, phytoceramide, dihydrophytoceramide, ceramine, dihydroceramine, phytoceramine, dihydrophytoceramine.
10. The use of Claim 9, wherein said sphingoid is a ceramide.
11. The use of any one of Claims 1 to 10, wherein said one or more
25 polyalkylamine chains are independently selected from spermine, spermidine, a polyalkylamine analog or a combination thereof.
12. The use of any one of Claims 1 to 11, wherein said target cell is a tissue.
13. The use of any one of Claims 1 to 12, in combination with one or more targeting substances.

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14. The use of any one of Claims 1 to 13, wherein said sphingoid-polyalkylamine conjugate is N-palmitoyl D-erythro sphingosyl carbamoyl-spermine (CCS).
15. A method for transfecting a nucleic acid into a target cell, said method comprises contacting said target cell with a sphingoid-polyalkylamine conjugate together with a nucleic acid molecule wherein said sphingoid-polyalkylamine conjugate comprises a sphingoid backbone carrying, via a carbamoyl bond, at least one polyalkylamine, thereby transfecting said target cell with the nucleic acid molecule.
16. The method of Claim 15, wherein said nucleic acid is associated with said sphingoid- polyalkylamine conjugate.
17. The method of Claim 16, wherein said nucleic acid molecule is a plasmid DNA.
18. The method of Claim 16, wherein said nucleic acid molecule is a small interference RNA (siRNA).
19. The method of Claim 16, wherein said nucleic acid molecule is an oligodeoxynucleotide (ODN).
20. The method of Claim 19, wherein said ODN contains at least one CpG motif (CpG-ODN).
21. The method of any one of Claims 15 to 20, wherein said sphingoid-polyalkylamine conjugate forms lipid assemblies.
22. The method of Claim 21 wherein said sphingoid-polyalkylamine conjugate forms vesicles and/or micelles.
23. The method of any one of Claims 15 to 22, wherein the sphingoid backbone is selected from ceramide, dihydroceramide, phytoceramide, dihydrophytoceramide, ceramine, dihydroceramine, phytoceramine, dihydrophytoceramine.
24. The method of Claim 23, wherein said sphingoid is a ceramide.

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25. The method of any one of Claims 15 to 24, wherein said one or more polyalkylamine chains are independently selected from spermine, spermidine, a polyalkylamine analog or a combination thereof.
26. The method of any one of Claims 15 to 25, wherein said target cell is a tissue.
27. The method of any one of Claims 15 to 26, wherein said sphingoid-polyalkylamine conjugate associated with the nucleic acid molecule is also associated with one or more targeting substances.
28. The method of any one of Claims 15 to 27, wherein said sphingoid-polyalkylamine conjugate is N-palmitoyl D-erythro sphingosyl carbamoyl-spermine (CCS).
29. A pharmaceutical composition for transfecting a nucleic acid into a target cell, the composition comprises: (i) at least one sphingoid- polyalkylamine conjugate, said sphingoid- polyalkylamine conjugate comprises a sphingoid backbone carrying, via a carbamoyl bond, at least one polyalkylamine chains; and (ii) at least one nucleic acid molecule associated with said conjugate.
30. The composition of Claim 29, comprising a physiologically acceptable carrier.
31. The composition of Claim 29 or 30, wherein said nucleic acid molecule has, at a physiological pH, a net negative dipole moment, at least one area carrying a negative charge or a net negative charge.
32. The composition of Claim 31, wherein said nucleic acid molecule is a plasmid DNA.
33. The composition of Claim 31, wherein said nucleic acid molecule is a small interference RNA (siRNA).
34. The composition of Claim 31, wherein said nucleic acid molecule is an oligodeoxynucleotide (ODN).
35. The composition of Claim 34, wherein said ODN contains at least one CpG motif (CpG-ODN).

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36. The composition of any one of Claims 29 to 35, wherein the sphingoid-polyalkylamine conjugate forms lipid assemblies.
37. The composition of Claim 36, wherein the sphingoid-polyalkylamine conjugate forms vesicles and/or micelles.
- 5 38. The composition of any one of Claims 29 to 37, wherein the sphingoid backbone is selected from ceramide, dihydroceramide, phytoceramide, dihydriophytoceramide, ceramine, dihydroceramine, phytoceramine, dihydriophytoceramine.
39. The composition of Claim 38, wherein said sphingoid is a ceramide.
- 10 40. The composition of any one of Claims 29 to 38, wherein said one or more polyalkylamine chains are independently selected from spermine, spermidine, a polyalkylamine analog or a combination thereof.
41. The composition of any one of Claims 29 to 40, wherein said target cell is a tissue.
- 15 42. The composition of any one of Claims 29 to 41, comprising one or more targeting substances.
43. The composition of any one of Claims 29 to 42, wherein said sphingoid-polyalkylamine conjugate is N-palmitoyl D-erythro sphingosyl carbamoyl-spermine (CCS).
- 20 44. A method for the treatment of a disease or disorder, the method comprises providing a subject in need of said treatment an amount of a sphingoid-polyalkylamine conjugate associated with a nucleic acid molecule, the amount being effective to achieve transfection of a target cell with said nucleic acid molecule and to achieve a desired biochemical effect on said target cell.
- 25 45. The method of Claim 44, wherein said nucleic acid is associated with said sphingoid- polyalkylamine conjugate.
46. The method of Claim 44 or 45, wherein said nucleic acid molecule is a plasmid DNA.
47. The method of Claim 44 or 45, wherein said nucleic acid molecule is a small
- 30 30 interference RNA (siRNA).

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48. The method of Claim 44 or 45, wherein said nucleic acid molecule is an oligodeoxynucleotide (ODN).
49. The method of Claim 48, wherein said ODN contains at least one CpG motif (CpG-ODN).
50. The method of any one of Claims 44 to 49, wherein said sphingoid-polyalkylamine conjugate forms lipid assemblies.
51. The method of Claim 50, wherein said sphingoid- polyalkylamine conjugates forms vesicles and/or micelles.
52. The method of any one of Claims 44 to 51, wherein the sphingoid backbone is selected from ceramide, dihydroceramide, phytoceramide, dihydrophytoceramide.
53. The method of Claim 52, wherein said sphingoid is a ceramide.
54. The method of any one of Claims 44 to 53, wherein said one or more polyalkylamine chains are independently selected from spermine, spermidine, a polyalkylamine analog or a combination thereof.
55. The method of any one of Claims 44 to 54, wherein said target cell is a tissue.
56. The method of any one of Claims 44 to 55, wherein said treatment includes ex vivo treatment of target cells with said sphingoid- polyalkylamine conjugate associated with a nucleic acid molecule, wherein said target cells are withdrawn from a subjects' body, and after treatment with said sphingoid- polyalkylamine conjugate associated with a nucleic acid molecule, the treated target cells are returned into the subject body.
57. The method of Claim 56, wherein said target cells are bone marrow cells.
58. The method of any one of Claims 44 to 57, wherein said sphingoid-polyalkylamine conjugate associated with the nucleic acid molecule is associated with one or more targeting substances.
59. The method of any one of Claims 44 to 58, wherein said sphingoid-polyalkylamine conjugate is N-palmitoyl D-erythro sphingosyl carbamoyl-spermine (CCS).

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60. A kit comprising a sphingoid-polyalkylamine conjugate as defined in any one of Claims 1 to 14, and instructions for use of said conjugate as a capturing agent of nucleic acid molecules.